CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

California Institute for Regenerative Medicine
Strategic Planning Advisory Committee
May 1, 2006

The first meeting of the Strategic Planning Advisory Committee (SPAC) was an introductory meeting at which the process for developing the CIRM strategic plan was discussed at length. In the course of this discussion, a number of ideas arose which are summarized below. This summary is intended to be comprehensive with respect to reporting these ideas; inclusion in this list does not imply any commitment or endorsement by the CIRM.

A. General Principles for the Scientific Strategic Plan

1. The scientific plan must be flexible, adaptable, and changeable.
   a. The plan must be able to adjust to both good and bad outcomes.
   b. The CIRM must have mechanisms in place to update its strategy based on these outcomes.

2. The plan should also consider the effects of possible (positive) changes in the future political climate.
   a. The CIRM should consider building a model that can leverage funding from the federal government if such changes occur.

B. Soliciting Grant Applications

1. CIRM needs to find more aggressive ways to engage and inform scientists about stem cell funding opportunities than sending out grant requests through the CIRM web site or the standard Request for Application (RFA) process. A newsletter might be a one way to accomplish this; to keep the "buzz" alive and to distribute information.

2. Conducting small meetings of scientists from unrelated disciplines is also good way to generate ideas for RFAs; the Pediatric AIDS Foundation and Hereditary Disease Foundation have used this approach effectively.

C. Strategic Considerations

1. The strategic plan needs to balance risky, innovative research with "low hanging fruit", that is, research that will yield immediate results with a minimum of risk involved.
   a. Identification of "low hanging fruit" over the next few years will allow CIRM to develop the mechanisms to transition CIRM sponsored research to the private sector (over the next 10 years and thereafter) so there is continuity when the mandate of Proposition 71 expires.
2. CIRM will need a good IP policy to assist with this transition to the private sector as well as commercialization efforts; an IP Task Force has been assigned to this key strategic task.
   a. The CIRM might consider bringing in an IP lawyer who has supported commercial enterprises and has a broad perspective.
   b. The CIRM should consider speaking with a person from NIH who is familiar with IP policy and grant administration.
   c. The CIRM should consider how IP policy (for non-profits) could restrict distribution of information and materials (reagents, etc.) to within California.

D. Possible Areas of Research Focus

1. General principles
   a. The CIRM must answer the question as to what extent it can or should fund research that is also funded by the NIH.
      i. The CIRM's initial priority should be to give preference to research currently not funded by the federal government.
      ii. NIH is facing financial hard times.

2. Cord blood research
   a. $100M has been set aside by the federal government for development of a cord blood bank.
      i. CIRM will need to determine whether cord blood research is therefore really an underfunded area with promise.
   b. There may be an opportunity at Children's Hospital Oakland Research Institute (CHORI), which focuses on using cord blood from a healthy newborn for transplantation to help a sibling with a blood disorder.
   c. There may be an opportunity to establish a California Cord Blood Bank.
      i. In the short-term, collecting cord blood, typing it for histocompatibility, and banking it could have a big impact.
      ii. Such a blood bank might be built through harvesting cord blood from directed donors (that is, by targeting members of a family where a disease that might be treated by cord blood transplantation already exists) rather than from all donors in order to capture the needs of California's large minority populations. This would be an effective way to distinguish the California effort.

d. Research into expansion of cord blood stem cell populations to enable treatment of larger children and adults as an alternative to bone marrow transplant might also be an area of focus.
    i. Cord blood transplantation is the accepted standard of care as an alternative to bone marrow transplant for children under 20 kg.

3. Tissue procurement
    a. Harvesting tissues from cadavers for different medical needs might also be a focus area.
    b. This field has experienced controversy in recent months, but it is critical to endeavors such as islet tissue transplantation.

4. Cell isolation (small and large scale)
    a. Fluorescent Activated Cell Sorting (or FACS) technology is currently inadequate for the large scale isolation of human cells.
    b. Researchers are working on a nano-scale approach to cell sorting that can be used for clinical applications.

5. Development of new stem cell lines
    a. The creation of disease specific lines using embryos captured from IVF might also be a focus area.
        i. Researchers at the Reproductive Institute of Chicago has 12-15 cell lines with single gene defects; these lines are valuable to the community interested in rare diseases.
        ii. The greatest value of research in this area may lie in improving the efficiency and methodology of cell line derivation.
    b. This type of work <u>cannot</u> be funded through NIH.

6. High-speed computing
    a. CIRM might consider investigating the use of high-speed computers for stem cell applications; many are already in use for genetic applications.
    b. California has access to large scale computing assets in the form of SUN Microsystems.
        i. SUN has a new generation of computers that are 10-100 times faster than some of the fastest ones currently available.
        ii. CIRM should consider looking at such strategic interfaces.

E. Infrastructure and Research Facilities

1. CIRM should also look into the need for a trained workforce and infrastructure requirements to commercialize and deliver on the research it sponsors.

2. Given the near term political climate, there is a great need to build stem cell research facilities or develop other innovative solutions to enable “non-Presidential” research.
    a. CIRM is authorized by Proposition 71 to spend 10% on facilities.

b. Facilities will mostly likely need to be built early in the 10-year timeframe established for Proposition 71.

3. An in depth discussion should be initiated with the Facilities Working Group and also consider speaking with an architect from the Howard Hughes Medical Institute, an architect who focuses on research facilities.

F. Collaboration / Cooperation

1. The strategic plan needs to address ways to cooperate with other states and countries compatible with Proposition 71.
   a. There could be great value in such cooperation as it would help identify assets the CIRM could leverage, including international assets that would help with clinical trials.

2. The CIRM should explore types of partnerships and collaborations (including interstate and international) allowed by Proposition 71 that are attractive from a scientific perspective and would serve to maintain California's reputation as a good scientific citizen with the national and international scientific communities.